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December 21, 2023

Dockets Management Staff (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

Re: **Docket No. 2023-20592** "Formal Meetings Between the FDA and Sponsors or Applicants of PDUFA Products"

To Whom it May Concern,

On behalf of patients and families impacted by rare diseases, the EveryLife Foundation for Rare Diseases is pleased to offer the following comments to inform the U.S. Food and Drug Administration on the Draft Guidance document entitled Formal Meetings Between the FDA and Sponsors or Applicants of PDUFA Products.

The EveryLife Foundation for Rare Diseases is a 501 (c)(3) nonprofit, nonpartisan organization dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments, and cures. EveryLife's establishment of a diverse coalition comprised of patient advocacy organizations, industry leaders, coalition groups, and other relevant stakeholders guides our policy efforts and provides advice and insight on urgent policy issues impacting the rare disease community.

As collaborative stakeholders, we have experienced a tremendous evolution of patient engagement and related areas since their infancy in PDUFA V -- to their continued development via 21st Century Cures and PDUFA VI -- and now their continued maturation today through the implementation of PDUFA VII. In this time, the landscape has shifted as the patient focused drug development movement evolved for the rare disease community. We've seen a deepened engagement and understanding of the patient perspective via numerous approaches such as the Patient Focused Drug Development meetings and the development of the FDA Benefit Risk Framework. We've had increased opportunities for meaningful participation in formal service on advisory committees, seen the formation of rare disease focused initiatives within CDER and CBER, seen the establishment of patient engagement advisory committees, and have benefited from reporting on the use of patient experience data within the regulatory review.

The PFDD Guidance Series, along with FDA's issuance of numerous other guidances that are informing the inclusion of patient experience data within clinical trial and patient-focused product development activities for drugs are resulting in lives changed and saved through products approved to treat rare conditions, especially those that have benefited from the inclusion of more

robust patient insights and patient experience data as part of the review process. By instituting a structured approach to listen to the voice of the patient in a meaningful way, the FDA has demonstrated its commitment to science and to ensuring appropriate processes are in place to quantify the perspective of the patient or the caregiver.

The implementation of PDUFA VII marks a significant milestone that should be celebrated by all who have devoted countless hours to the work of integrating patient voices into the therapy development ecosystem. We are grateful for the opportunity to engage in the innovations that may enable us to more fully realize the potential of PFDD efforts through greater incorporation of patient experience data in regulatory decision making.

Recognizing the growth and evolution of the PFDD field, the EveryLife Foundation led The PFDD Rare Disease Guidance Compendium Workshop Series in 2021 in collaboration with the National Health Council, BIO, PhRMA and 88 subject matter experts from across the rare disease ecosystem. The Workshop Series explored patient engagement practices throughout the medical product development process, from early-stage research through post-market integration into healthcare, and assessed 20 FDA Guidances and 112 regulatory development resources informing the inclusion of patient experience data in therapy development. The Workshop series and assessments yielded the publication of the "Guide to Patient Involvement in Rare Disease Therapy Development in 2022."

As the field evolves and more patient organizations, researchers and sponsors invest in the collection and use of patient experience data, one of the most common challenges raised in the Workshop Series and within subsequent convenings of rare disease community experts has been the lack of opportunities to interact with the agency about patient experience data collection plans and other patient engagement related activities. Sponsors and other stakeholders invested in the collection and use of patient experience data believe such avenues for communication with the agency are critical to improving the chances this information will be used in decision-making.

The rare disease community applauds the establishment of the newly established Type D meetings and offer the following suggestions to optimize the meeting format to address some of the early communication needs expressed by stakeholders.

Explicitly reference 'patient experience' and 'patient focused drug development (PFDD)' content as topic eligible for Type D meetings, to include joint meetings involving patient organizations and/or research partners involved in collecting and synthesizing such data.

One measurable way to increase such opportunities would occur by amending the draft guidance such that it explicitly references patient experience and patient-focused drug development (PFDD) content as a topic eligible for Type D meetings, including joint meetings involving patient organizations and/or research partners involved in collecting and synthesizing such data. For sponsors and stakeholders to more fully embrace the use of patient engagement tools it is essential that high-quality and rigorous patient engagement studies and tools be developed. This goal is not achievable absent guidance from the FDA, particularly from product reviewers.

An explicit reference to patient engagement or patient experience data within the description of Type D meetings will help clarify and confirm this point. Specifically, we submit the following example for the agency's consideration for addition to the draft guidance's list of the three issues that requests can include:

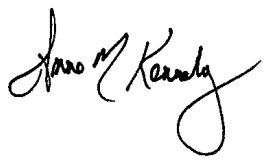
> *An issue pertaining to the collection of patient experience data, such as information from risk-benefit studies, patient reported outcomes tools, patient preference studies, and similar data for the possible inclusion as part of a product application.*

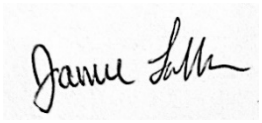
Further, we recommend the inclusion of at least one PFDD-related scenario in the list of Type D meeting 'Examples and Scenarios' detailed in the draft guidance.

While Type D meetings are focused on narrow topics, there is a need for engagement in the pre-competitive space that looks at a larger range of topics including initiatives looking at endpoints that are meaningful to patients, lived experiences with the condition, and how patient experience views can ultimately be reflected within product labels. While we recognize the important role of externally led PFDD meetings in accomplishing this goal, rare disease stakeholders have expressed a need for additional mechanisms that involve more focused dialogue and can occur as the science or therapy development landscape evolves over time.

The EveryLife Foundation looks forward to giving additional thought to the evolution of the PFDD field over the months and years ahead and welcomes the opportunity to engage directly with agency leaders on it as we all work to refine and mature this field.

Thank you for the opportunity to provide comments and for your ongoing commitment to addressing the unmet needs of the rare disease population. If we can provide additional information to support your process, please contact Annie Kennedy, Chief of Policy, Advocacy and Patient Engagement akennedy@everylifefoundation.org.





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